



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2017-N-0809]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (the FD&C Act), as amended by the Food and Drug Administration Safety and Innovation Act (FDASIA), authorizes FDA to award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA is required to publish notice of the award of the priority review voucher. FDA has determined that KYMRIA[®] (tisagenlecleucel), manufactured by Novartis Pharmaceuticals Corporation, meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Gretchen Opper, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911.

SUPPLEMENTARY INFORMATION: FDA is announcing the issuance of a priority review voucher to the sponsor of an approved rare pediatric disease product application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), which was added by FDASIA, FDA will award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA has determined that KYMRIA[®] (tisagenlecleucel), manufactured by

Novartis Pharmaceuticals Corporation, meets the criteria for a priority review voucher.

KYMRIAH (tisagenlecleucel) is indicated for the treatment of patients up to 25 years of age with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse.

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to <https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm>. For further information about KYMRIAH (tisagenlecleucel), go to the Center for Biologics Evaluation and Research cellular and gene therapy products website at <https://www.fda.gov/BiologicsBloodVaccines/CellularGeneTherapyProducts/ApprovedProducts/default.htm>.

Dated: September 5, 2017.

Anna K. Abram,

Deputy Commissioner for Policy, Planning, Legislation, and Analysis.

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